Board Funds Fifteen Bridges to Stem Cell Research and Therapy Programs Across California and New Sickle Cell Disease Trial

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Oakland, CA – Today the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded $8.39 million to the University of California, San Francisco (UCSF) to fund a clinical trial for sickle cell disease (SCD). An additional $51.08 million was awarded to fifteen community colleges and universities across California to fund undergraduate and master's level programs that will help train the next generation of stem cell researchers.

SCD is an inherited blood disorder caused by a single gene mutation that changes a single base in the B globin gene leading to the production of defective hemoglobin that polymerizes and damages red blood cells thus the "sickle" shaped red blood cells. The damaged cells cause blood vessels to occlude/close up and that can lead to multiple organ damage as well as reduced quality of life and life expectancy.

Mark Walters, M.D., and his team at UCSF Benioff Children's Hospital Oakland will be conducting a clinical trial that uses CRISPR-Cas9 gene editing technology to correct the genetic mutation in the blood stem cells of patients with severe SCD. The corrected blood stem cells will then be reintroduced back into patients with the goal of correcting the defective hemoglobin and thus producing functional, normal shaped red blood cells.

This clinical trial will be eligible for co-funding under the landmark agreement between CIRM and the National Heart, Lung, and Blood Institute (NHLBI) of the NIH. The CIRM-NHLBI agreement is intended to co-fund cell and gene therapy programs under the NHLBI's "Cure Sickle Cell" initiative. The goal is to markedly accelerate the development of cell and gene therapies for SCD. CIRM has previously funded the preclinical development of this therapy through a Translational award as well as its IND-enabling studies through a Late Stage Preclinical award in partnership with NHLBI.

The CIRM Bridges to Stem Cell Research and Therapy program provides undergraduate and master's students with the opportunity to take stem cell related courses and receive hands on experience and training in a stem cell research related laboratory at a university or biotechnology company. Fifteen institutions received a total of $51.08 million to carry out these programs to train the next generation of scientists.

The awards are summarized in the table below.

Application	Title	Institution	Award Amount
EDUC2-12607	Bridges to Stem Cell Research and Therapy at Pasadena City College	Pasadena City College	$3,605,500
EDUC2-12611	CIRM Bridges to Stem Cell Research and Therapy Training Grant	CSU San Marcos	$3,606,500
EDUC2-12617	Bridges to Stem Cell Research Internship Program	San Diego State University	$3,605,500

EDUC2-12620	CIRM Bridges 3.0	Humboldt State	$3,605,495
EDUC2-12638	CIRM Regenerative Medicine and Stem Cell Research Biotechnology Training Program	CSU Long Beach	$3,276,500
EDUC2-12677	Stem Cell Internships in Laboratory-based Learning (SCILL) continue to expand the scientific workforce for stem cells research and therapies.	San Jose State University	$3,605,500
EDUC2-12691	Strengthening the Pipeline of Master's-level Scientific and Laboratory Personnel in Stem Cell Research	CSU Sacramento	$2,946,500
EDUC2-12693	CIRM Bridges Science Master's Program	San Francisco State University	$3,606,500
EDUC2-12695	CIRM Graduate Student Training in Stem Cell Sciences in the Stem Cell Technology and Lab Management Emphasis of the MS Biotechnology Program	CSU Channel Islands	$3,606,500
EDUC2-12718	CSUN CIRM Bridges 3.0 Stem Cell Research & Therapy Training Program	CSU Northridge	$3,606,500
EDUC2-12720	Stem Cell Scholars: a workforce development pipeline, educating, training and engaging students from basic research to clinical translation.	CSU San Bernardino	$3,606,500
EDUC2-12726	Training Master's Students to Advance the Regenerative Medicine Field	Cal Poly San Luis Obispo	$3,276,500
EDUC2-12730	Building Career Pathways into Stem Cell Research and Therapy Development	City College of San Francisco	$2,706,200

EDUC2-12734	Bridges to Stem Cell Research and Therapy: A Talent Development Program for Training Diverse Undergraduates for Careers in Regenerative Medicine	CSU Fullerton	$3,606,500
EDUC2-12738	CIRM Bridges to Stem Cell Research and Therapy	Berkeley City College	$2,806,896

“We are pleased to fund a promising trial for sickle cell disease that uses the Nobel Prize winning gene editing technology CRISPR-Cas9,” says Maria T. Millan, M.D., President and CEO of CIRM. “This clinical trial is a testament to how the CIRM model supports promising early-stage research, accelerates it through translational development, and advances it into the clinics. As the field advances, we must also meet the demand for promising young scientists. The CIRM Bridges programs across the state of California will provide students with the tools and resources to begin their careers in regenerative medicine.”

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today’s most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world’s largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov
